March 11, 2022

The Lancet Publishes Positive Results from Capricor Therapeutics' Phase 2 Study Evaluating CAP-1002 in Late-Stage Duchenne Muscular Dystrophy

-One-Year Final Data Highlights Lead Cell Therapy Asset, CAP-1002, for Safety and Efficacy in Slowing Upper Limb and Cardiac Function Deterioration in Patients-

-Data Sets Stage for Pivotal Phase 3 HOPE-3 Clinical Trial-

SAN DIEGO, March 11, 2022 (GLOBE NEWSWIRE) -- Capricor Therapeutics (NASDAQ: CAPR), a biotechnology company focused on the development of transformative cell and exosome-based therapeutics for the treatment and prevention of a broad spectrum of diseases, announced today that *The Lancet*, a renowned peer-reviewed global medical source for clinical and global health, has published positive results from Capricor's HOPE-2 Phase 2 clinical trial using lead asset, CAP-1002, to treat patients in advanced stages of Duchenne muscular dystrophy (DMD). Specifically, the study demonstrated that young men in the advanced stages of DMD experienced improvements in both skeletal and cardiac muscle function after receiving four doses of CAP-1002 over the course of one year. The paper titled, "*Repeated intravenous cardiosphere-derived cell therapy in late-stage Duchenne muscular dystrophy (HOPE-2): a multicentre, randomised, double-blind, placebo-controlled, phase 2 trial*," can be accessed here.

"Robust research is imperative to truly usher in a new class of treatments for these patients in dire need and historically Duchenne trials have not focused of the most severely affected non-ambulatory patients with the greatest disease burden," said Dr. Craig McDonald, the national principal investigator for HOPE-2, UC Davis professor and chair of the Department of Physical Medicine and Rehabilitation and lead author of the study. "We believe that the publication of these Phase 2 results in an eminent publication such as The Lancet, represents a major achievement for the development of CAP-1002 and further validates the power of this cell therapy for all patients with DMD regardless of their stage of disease."

"CAP-1002 has shown safety and very promising efficacy in patients with DMD in two successful clinical trials to date, setting the stage for a novel therapeutic that can be life changing for patients with DMD," said Linda Marbán, Ph.D., Chief Executive Officer of Capricor. "Further, our recently announced partnership with Nippon Shinyaku has secured us a commercialization and distribution partner in the US, experienced in rare diseases with specific expertise in DMD. Capricor is initiating our Phase 3, HOPE-3 clinical study imminently as we move towards potential commercialization."

HOPE-2 Study Details

HOPE-2 was a randomized, double-blind, placebo-controlled, Phase 2 clinical trial with the Company's lead investigational therapy, CAP-1002, in boys and young men who have DMD and are in the later stages of the disease's progression. Presently, this comprises approximately half of all DMD patients. The trial was conducted at nine sites across the United States. Study patients were treated via intravenous delivery with either CAP-1002 (150 million cells per infusion) or a placebo every three months. Data from a total of 20 patients was analyzed (12 placebo and eight treated) at the 12-month mark. Approximately 80 percent of patients were non-ambulant, and all patients were on a stable regimen of steroids. Demographic and baseline characteristics were similar between the two treatment groups.

Final data analysis demonstrated that young men in the advanced stages of DMD experienced improvements in skeletal and cardiac measurements after receiving four doses of CAP-1002 over the course of one year. Subjects in the trial were evaluated using the Performance of the Upper Limb (PUL), a validated tool specifically designed for assessing high (shoulder), mid (elbow) and distal (wrist and hand) function, with a conceptual framework reflecting the progression of weakness in upper limb function. CAP-1002 was generally safe and well-tolerated throughout the study. With the exception of hypersensitivity reactions early in the clinical trial, which were mitigated with a common pre-medication regimen, there were no serious safety signals identified by the HOPE-2 Data and Safety Monitoring Board (DSMB).

Pat Furlong, Founding President and CEO of Parent Project Muscular Dystrophy, a nonprofit organization leading the fight to end Duchenne said, "The results from this study are highly encouraging for people with Duchenne, especially our non-ambulatory community who have limited therapeutic options. Further, CAP-1002 has demonstrated cardiac benefits in this patient population where heart failure continues to be the leading cause of mortality. These data support the hope that CAP-1002 may one day become an important therapeutic option and slow the advancement of the disease."

About CAP-1002

CAP-1002 consists of allogeneic cardiosphere-derived cells, or CDCs, a type of progenitor cell that has been shown in pre-clinical and clinical studies to exert potent immunomodulatory activity and is being investigated for its potential to modify the immune system's activity to encourage cellular regeneration. CDCs have been the subject of over 100 peer-reviewed scientific publications and have been administered to over 200 patients across several clinical trials.

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy is a devastating genetic disorder characterized by progressive weakness and chronic inflammation of the skeletal, heart and respiratory muscles. Patients suffering from DMD typically lose their ability to walk in their teenage years and generally die of cardiac or respiratory complications by age 30. It occurs in one in every 3,600 live male births across all races, cultures and countries. DMD afflicts approximately 200,000 boys and young men around the world. Treatment options are limited, and there is no cure.

About Capricor Therapeutics

Capricor Therapeutics, Inc. (NASDAQ: CAPR) is a biotechnology company focused on developing transformative cell and exosome-based therapeutics and vaccines for treating and preventing a broad spectrum of diseases. Capricor's lead candidate, CAP-1002, is an allogeneic cardiac-derived cell therapy that is currently in clinical development for treating Duchenne muscular dystrophy and the cytokine storm associated with COVID-19. Capricor is also developing its exosome technology as a next-generation therapeutic platform. The Company's current focus is on developing exosomes capable of delivering nucleic acids, including mRNA as well as proteins, to treat or prevent a variety of diseases. For more information, visit www.capricor.com, and follow the Company on Facebook, Instagram and Twitter.

Cautionary Note Regarding Forward-Looking Statements

Statements in this press release regarding the efficacy, safety, and intended utilization of Capricor's product candidates; the initiation, conduct, size, timing and results of discovery efforts and clinical trials; the pace of enrollment of clinical trials; plans regarding regulatory filings, future research and clinical trials; regulatory developments involving products, including the ability to obtain regulatory approvals or otherwise bring products to market; the ability to achieve product milestones and to receive milestone payments from commercial partners; plans regarding current and future collaborative activities and the ownership of commercial rights; scope, duration, validity and enforceability of intellectual property rights; future royalty streams, revenue projections; expectations with respect to the expected use of proceeds from the recently completed offerings and the anticipated effects of the offerings; and any other statements about Capricor's management team's future expectations, beliefs, goals, plans or prospects constitute forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Any statements that are not statements of historical fact (including statements containing the words "believes," "plans," "could," "anticipates," "expects," "estimates," "should," "target," "will," "would" and similar expressions) should also be considered to be forward-looking statements. There are a number of important factors that could cause actual results or events to differ materially from those indicated by such forward-looking statements. More information about these and other risks that may impact Capricor's business is set forth in Capricor's Annual Report on Form 10-K for the year ended December 31, 2021, as filed with the Securities and Exchange Commission on March 11, 2022. All forward-looking statements in this press release are based on information available to Capricor as of the date hereof, and Capricor assumes no obligation to update these forward-looking statements.

CAP-1002 is an Investigational New Drug and is not approved for any indications. None of Capricor's exosome-based candidates have been approved for clinical investigation.

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Source: Capricor Therapeutics